<110> Hildinger, Markus

<120> Decreasing gene expression in a mammalian subject in vivo via
      AAV-mediated RNAi expression cassette transfer

<130> 1339

<140> US 10/604,340
<141> 2003-07-13

<160> 11

<170> PatentIn version 3.1

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      expression cassette; referred to as AAV2/2 CMV luciferase

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aac gtg caa aaa aag ctc cca atc atc caa aaa att att atc atg gat       1686
Asn Val Gln Lys Lys Leu Pro Ile Ile Gln Lys Ile Ile Ile Met Asp
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Thr Gly Leu Pro Lys Gly Val Ala Leu Pro His Arg Thr Ala Cys Val
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aga ttc tcg cat gcc aga gat cct att ttt ggc aat caa atc att ccg        1926
Arg Phe Ser His Ala Arg Asp Pro Ile Phe Gly Asn Gln Ile Ile Pro
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gat act gcg att tta agt gtt gtt cca ttc cat cac ggt ttt gga atg        1974
Asp Thr Ala Ile Leu Ser Val Val Pro Phe His His Gly Phe Gly Met
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att caa agt gcg ctg ctg gtg cca acc cta ttc tcc ttc ttc gcc aaa        2118
Ile Gln Ser Ala Leu Leu Val Pro Thr Leu Phe Ser Phe Phe Ala Lys
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agc act ctg att gac aaa tac gat tta tct aat tta cac gaa att gct        2166
Ser Thr Leu Ile Asp Lys Tyr Asp Leu Ser Asn Leu His Glu Ile Ala
            300                 305                 310

tct ggt ggc gct ccc ctc tct aag gaa gtc ggg gaa gcg gtt gcc aag        2214
Ser Gly Gly Ala Pro Leu Ser Lys Glu Val Gly Glu Ala Val Ala Lys
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Arg Phe His Leu Pro Gly Ile Arg Gln Gly Tyr Gly Leu Thr Glu Thr
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Val Gly Lys Val Val Pro Phe Phe Glu Ala Lys Val Val Asp Leu Asp
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acc ggg aaa acg ctg ggc gtt aat caa aga ggc gaa ctg tgt gtg aga        2406
Thr Gly Lys Thr Leu Gly Val Asn Gln Arg Gly Glu Leu Cys Val Arg
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ggt cct atg att atg tcc ggt tat gta aac aat ccg gaa gcg acc aac        2454
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gcc ttg att gac aag gat gga tgg cta cat tct gga gac ata gct tac        2502
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Tyr Ala Leu Val Pro Gly Thr Ile Ala Phe Thr Asp Ala His Ile Glu
        35                  40                  45


Val Asp Ile Thr Tyr Ala Glu Tyr Phe Glu Met Ser Val Arg Leu Ala
    50                  55                  60
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Glu Ala Met Lys Arg Tyr Gly Leu Asn Thr Asn His Arg Ile Val Val
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Cys Ser Glu Asn Ser Leu Gln Phe Phe Met Pro Val Leu Gly Ala Leu
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Phe Ile Gly Val Ala Val Ala Pro Ala Asn Asp Ile Tyr Asn Glu Arg
            100   105   110

Glu Leu Leu Asn Ser Met Gly Ile Ser Gln Pro Thr Val Val Phe Val
        115   120   125

Ser Lys Lys Gly Leu Gln Lys Ile Leu Asn Val Gln Lys Lys Leu Pro
    130   135   140

Ile Ile Gln Lys Ile Ile Ile Met Asp Ser Lys Thr Asp Tyr Gln Gly
145   150   155   160

Phe Gln Ser Met Tyr Thr Phe Val Thr Ser His Leu Pro Pro Gly Phe
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Asn Glu Tyr Asp Phe Val Pro Glu Ser Phe Asp Arg Asp Lys Thr Ile
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Ala Leu Ile Met Asn Ser Ser Gly Ser Thr Gly Leu Pro Lys Gly Val
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Ala Leu Pro His Arg Thr Ala Cys Val Arg Phe Ser His Ala Arg Asp
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Pro Ile Phe Gly Asn Gln Ile Ile Pro Asp Thr Ala Ile Leu Ser Val
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Val Pro Phe His His Gly Phe Gly Met Phe Thr Thr Leu Gly Tyr Leu
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Ile Cys Gly Phe Arg Val Val Leu Met Tyr Arg Phe Glu Glu Glu Leu
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Phe Leu Arg Ser Leu Gln Asp Tyr Lys Ile Gln Ser Ala Leu Leu Val
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Pro Thr Leu Phe Ser Phe Phe Ala Lys Ser Thr Leu Ile Asp Lys Tyr
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Asp Leu Ser Asn Leu His Glu Ile Ala Ser Gly Gly Ala Pro Leu Ser
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Lys Glu Val Gly Glu Ala Val Ala Lys Arg Phe His Leu Pro Gly Ile
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Arg Gln Gly Tyr Gly Leu Thr Glu Thr Thr Ser Ala Ile Leu Ile Thr
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Pro Glu Gly Asp Asp Lys Pro Gly Ala Val Gly Lys Val Val Pro Phe
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Phe Glu Ala Lys Val Val Asp Leu Asp Thr Gly Lys Thr Leu Gly Val
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Asn Gln Arg Gly Glu Leu Cys Val Arg Gly Pro Met Ile Met Ser Gly
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Tyr Val Asn Asn Pro Glu Ala Thr Asn Ala Leu Ile Asp Lys Asp Gly
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Trp Leu His Ser Gly Asp Ile Ala Tyr Trp Asp Glu Asp Glu His Phe
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Phe Ile Val Asp Arg Leu Lys Ser Leu Ile Lys Tyr Lys Gly Tyr Gln
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Val Ala Pro Ala Glu Leu Glu Ser Ile Leu Leu Gln His Pro Asn Ile
    450   455   460

Phe Asp Ala Gly Val Ala Gly Leu Pro Asp Asp Asp Ala Gly Glu Leu
465   470   475   480

Pro Ala Ala Val Val Val Leu Glu His Gly Lys Thr Met Thr Glu Lys
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Glu Ile Val Asp Tyr Val Ala Ser Gln Val Thr Thr Ala Lys Lys Leu
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Arg Gly Gly Val Val Phe Val Asp Glu Val Pro Lys Gly Leu Thr Gly
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                              1               5

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Gly Pro Ala Pro Phe Tyr Pro Leu Glu Asp Gly Thr Ala Gly Glu Gln
10              15              20              25

ctg cat aag gct atg aag aga tac gcc ctg gtt cct gga aca att gct       1350
Leu His Lys Ala Met Lys Arg Tyr Ala Leu Val Pro Gly Thr Ile Ala
            30              35              40

ttt aca gat gca cat atc gag gtg gac atc act tac gct gag tac ttc       1398
Phe Thr Asp Ala His Ile Glu Val Asp Ile Thr Tyr Ala Glu Tyr Phe
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Glu Met Ser Val Arg Leu Ala Glu Ala Met Lys Arg Tyr Gly Leu Asn
        60              65              70

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Thr Asn His Arg Ile Val Val Cys Ser Glu Asn Ser Leu Gln Phe Phe
        75              80              85

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Met Pro Val Leu Gly Ala Leu Phe Ile Gly Val Ala Val Ala Pro Ala
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            110             115             120

cag cct acc gtg gtg ttc gtt tcc aaa aag ggg ttg caa aaa att ttg       1638
Gln Pro Thr Val Val Phe Val Ser Lys Lys Gly Leu Gln Lys Ile Leu
            125             130             135

aac gtg caa aaa aag ctc cca atc atc caa aaa att att atc atg gat       1686
Asn Val Gln Lys Lys Leu Pro Ile Ile Gln Lys Ile Ile Ile Met Asp
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tct cat cta cct ccc ggt ttt aat gaa tac gat ttt gtg cca gag tcc       1782
Ser His Leu Pro Pro Gly Phe Asn Glu Tyr Asp Phe Val Pro Glu Ser
170             175             180             185

ttc gat agg gac aag aca att gca ctg atc atg aac tcc tct gga tct       1830
Phe Asp Arg Asp Lys Thr Ile Ala Leu Ile Met Asn Ser Ser Gly Ser
            190             195             200

act ggt ctg cct aaa ggt gtc gct ctg cct cat aga act gcc tgc gtg       1878
Thr Gly Leu Pro Lys Gly Val Ala Leu Pro His Arg Thr Ala Cys Val
            205             210             215

aga ttc tcg cat gcc aga gat cct att ttt ggc aat caa atc att ccg       1926
```

```
Arg Phe Ser His Ala Arg Asp Pro Ile Phe Gly Asn Gln Ile Ile Pro
        220             225                 230

gat act gcg att tta agt gtt gtt cca ttc cat cac ggt ttt gga atg     1974
Asp Thr Ala Ile Leu Ser Val Val Pro Phe His His Gly Phe Gly Met
235                 240                 245

ttt act aca ctc gga tat ttg ata tgt gga ttt cga gtc gtc tta atg     2022
Phe Thr Thr Leu Gly Tyr Leu Ile Cys Gly Phe Arg Val Val Leu Met
250                 255                 260                 265

tat aga ttt gaa gaa gag ctg ttt ctg agg agc ctt cag gat tac aag     2070
Tyr Arg Phe Glu Glu Glu Leu Phe Leu Arg Ser Leu Gln Asp Tyr Lys
                270                 275                 280

att caa agt gcg ctg ctg gtg cca acc cta ttc tcc ttc ttc gcc aaa     2118
Ile Gln Ser Ala Leu Leu Val Pro Thr Leu Phe Ser Phe Phe Ala Lys
                285                 290                 295

agc act ctg att gac aaa tac gat tta tct aat tta cac gaa att gct     2166
Ser Thr Leu Ile Asp Lys Tyr Asp Leu Ser Asn Leu His Glu Ile Ala
            300                 305                 310

tct ggt ggc gct ccc ctc tct aag gaa gtc ggg gaa gcg gtt gcc aag     2214
Ser Gly Gly Ala Pro Leu Ser Lys Glu Val Gly Glu Ala Val Ala Lys
315                 320                 325

agg ttc cat ctg cca ggt atc agg caa gga tat ggg ctc act gag act     2262
Arg Phe His Leu Pro Gly Ile Arg Gln Gly Tyr Gly Leu Thr Glu Thr
330                 335                 340                 345

aca tca gct att ctg att aca ccc gag ggg gat gat aaa ccg ggc gcg     2310
Thr Ser Ala Ile Leu Ile Thr Pro Glu Gly Asp Asp Lys Pro Gly Ala
                350                 355                 360

gtc ggt aaa gtt gtt cca ttt ttt gaa gcg aag gtt gtg gat ctg gat     2358
Val Gly Lys Val Val Pro Phe Phe Glu Ala Lys Val Val Asp Leu Asp
            365                 370                 375

acc ggg aaa acg ctg ggc gtt aat caa aga ggc gaa ctg tgt gtg aga     2406
Thr Gly Lys Thr Leu Gly Val Asn Gln Arg Gly Glu Leu Cys Val Arg
            380                 385                 390

ggt cct atg att atg tcc ggt tat gta aac aat ccg gaa gcg acc aac     2454
Gly Pro Met Ile Met Ser Gly Tyr Val Asn Asn Pro Glu Ala Thr Asn
        395                 400                 405

gcc ttg att gac aag gat gga tgg cta cat tct gga gac ata gct tac     2502
Ala Leu Ile Asp Lys Asp Gly Trp Leu His Ser Gly Asp Ile Ala Tyr
410                 415                 420                 425

tgg gac gaa gac gaa cac ttc ttc atc gtt gac cgc ctg aag tct ctg     2550
Trp Asp Glu Asp Glu His Phe Phe Ile Val Asp Arg Leu Lys Ser Leu
                430                 435                 440

att aag tac aaa ggc tat cag gtg gct ccc gct gaa ttg gaa tcc atc     2598
Ile Lys Tyr Lys Gly Tyr Gln Val Ala Pro Ala Glu Leu Glu Ser Ile
                445                 450                 455

ttg ctc caa cac ccc aac atc ttc gac gca ggt gtc gca ggt ctt ccc     2646
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Leu Leu Gln His Pro Asn Ile Phe Asp Ala Gly Val Ala Gly Leu Pro
        460             465             470

gac gat gac gcc ggt gaa ctt ccc gcc gcc gtt gtt gtt ttg gag cac    2694
Asp Asp Asp Ala Gly Glu Leu Pro Ala Ala Val Val Val Leu Glu His
        475             480             485

gga aag acg atg acg gaa aaa gag atc gtg gat tac gtc gcc agt caa    2742
Gly Lys Thr Met Thr Glu Lys Glu Ile Val Asp Tyr Val Ala Ser Gln
490             495             500             505

gta aca acc gcg aaa aag ttg cgc gga gga gtt gtg ttt gtg gac gaa    2790
Val Thr Thr Ala Lys Lys Leu Arg Gly Gly Val Val Phe Val Asp Glu
            510             515             520

gta ccg aaa ggt ctt acc gga aaa ctc gac gca aga aaa atc aga gag    2838
Val Pro Lys Gly Leu Thr Gly Lys Leu Asp Ala Arg Lys Ile Arg Glu
            525             530             535

atc ctc ata aag gcc aag aag ggc gga aag atc gcc gtg taa taa        2883
Ile Leu Ile Lys Ala Lys Lys Gly Gly Lys Ile Ala Val
        540             545             550

gcttggatcc aatcaacctc tggattacaa aatttgtgaa agattgactg gtattcttaa    2943

ctatgttgct ccttttacgc tatgtggata cgctgcttta atgcctttgt atcatgctat    3003

tgcttcccgt atggctttca ttttctcctc cttgtataaa tcctggttgc tgtctcttta    3063

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tccccctttc gccagctggc gtaatagcga agaggcccgc accgatcgcc cttcccaaca    4023
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cgtagaaaag atcaaaggat cttcttgaga tcctttttttt ctgcgcgtaa tctgctgctt    5703

gcaaacaaaa aaaccaccgc taccagcggt ggtttgtttg ccggatcaag agctaccaac    5763

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cggaacagga gagcgcacga gggagcttcc aggggggaaac gcctggtatc tttatagtcc    6183

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ttttgctcac atgttctttc ctgcgttatc ccctgattct gtggataacc gtattaccgc    6363

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<212> PRT
<213> Artificial

<220>
<223> sequence for recombinant adeno-associated viral vector, including
      plasmid backbone, with AAV2 internal terminal repeats that flank
      expression cassette; referred to as AAV2/5 CMV luciferase

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Leu Glu Asp Gly Thr Ala Gly Glu Gln Leu His Lys Ala Met Lys Arg
            20                  25                  30


Tyr Ala Leu Val Pro Gly Thr Ile Ala Phe Thr Asp Ala His Ile Glu
        35                  40                  45


Val Asp Ile Thr Tyr Ala Glu Tyr Phe Glu Met Ser Val Arg Leu Ala
    50                  55                  60


Glu Ala Met Lys Arg Tyr Gly Leu Asn Thr Asn His Arg Ile Val Val
65                  70                  75                  80


Cys Ser Glu Asn Ser Leu Gln Phe Phe Met Pro Val Leu Gly Ala Leu
                85                  90                  95
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```

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Phe Ile Gly Val Ala Val Ala Pro Ala Asn Asp Ile Tyr Asn Glu Arg
            100                 105                 110

Glu Leu Leu Asn Ser Met Gly Ile Ser Gln Pro Thr Val Val Phe Val
            115                 120                 125

Ser Lys Lys Gly Leu Gln Lys Ile Leu Asn Val Gln Lys Lys Leu Pro
            130                 135                 140

Ile Ile Gln Lys Ile Ile Ile Met Asp Ser Lys Thr Asp Tyr Gln Gly
145                 150                 155                 160

Phe Gln Ser Met Tyr Thr Phe Val Thr Ser His Leu Pro Pro Gly Phe
                165                 170                 175

Asn Glu Tyr Asp Phe Val Pro Glu Ser Phe Asp Arg Asp Lys Thr Ile
                180                 185                 190

Ala Leu Ile Met Asn Ser Ser Gly Ser Thr Gly Leu Pro Lys Gly Val
                195                 200                 205

Ala Leu Pro His Arg Thr Ala Cys Val Arg Phe Ser His Ala Arg Asp
            210                 215                 220

Pro Ile Phe Gly Asn Gln Ile Ile Pro Asp Thr Ala Ile Leu Ser Val
225                 230                 235                 240

Val Pro Phe His His Gly Phe Gly Met Phe Thr Thr Leu Gly Tyr Leu
                245                 250                 255

Ile Cys Gly Phe Arg Val Val Leu Met Tyr Arg Phe Glu Glu Glu Leu
                260                 265                 270

Phe Leu Arg Ser Leu Gln Asp Tyr Lys Ile Gln Ser Ala Leu Leu Val
            275                 280                 285

Pro Thr Leu Phe Ser Phe Phe Ala Lys Ser Thr Leu Ile Asp Lys Tyr
290                 295                 300

Asp Leu Ser Asn Leu His Glu Ile Ala Ser Gly Gly Ala Pro Leu Ser
305                 310                 315                 320

Lys Glu Val Gly Glu Ala Val Ala Lys Arg Phe His Leu Pro Gly Ile
                325                 330                 335
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Arg Gln Gly Tyr Gly Leu Thr Glu Thr Thr Ser Ala Ile Leu Ile Thr
            340                 345                 350

Pro Glu Gly Asp Asp Lys Pro Gly Ala Val Gly Lys Val Val Pro Phe
            355                 360                 365

Phe Glu Ala Lys Val Val Asp Leu Asp Thr Gly Lys Thr Leu Gly Val
    370                 375                 380

Asn Gln Arg Gly Glu Leu Cys Val Arg Gly Pro Met Ile Met Ser Gly
385                 390                 395                 400

Tyr Val Asn Asn Pro Glu Ala Thr Asn Ala Leu Ile Asp Lys Asp Gly
                405                 410                 415

Trp Leu His Ser Gly Asp Ile Ala Tyr Trp Asp Glu Asp Glu His Phe
            420                 425                 430

Phe Ile Val Asp Arg Leu Lys Ser Leu Ile Lys Tyr Lys Gly Tyr Gln
            435                 440                 445

Val Ala Pro Ala Glu Leu Glu Ser Ile Leu Leu Gln His Pro Asn Ile
    450                 455                 460

Phe Asp Ala Gly Val Ala Gly Leu Pro Asp Asp Asp Ala Gly Glu Leu
465                 470                 475                 480

Pro Ala Ala Val Val Val Leu Glu His Gly Lys Thr Met Thr Glu Lys
                485                 490                 495

Glu Ile Val Asp Tyr Val Ala Ser Gln Val Thr Thr Ala Lys Lys Leu
            500                 505                 510

Arg Gly Gly Val Val Phe Val Asp Glu Val Pro Lys Gly Leu Thr Gly
            515                 520                 525

Lys Leu Asp Ala Arg Lys Ile Arg Glu Ile Leu Ile Lys Ala Lys Lys
    530                 535                 540

Gly Gly Lys Ile Ala Val
545                 550


<210>   5
<211>   3618
<212>   DNA
<213>   Artificial
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<220>
<223> sequence for recombinant adeno-associated viral vector, including
       plasmid backbone, with AAV2 internal terminal repeats that flank
       expression cassette; referred to as AAV2/5 U6 lucRI-1a

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ttgtgagcgg ataacaattt cacacaggaa acagctatga ccatgattac gccagattta   240

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catcactagg ggttccttgt agttaatgat taacccgcca tgctacttat ctacgtagcc   420

atgctctagg aagatcggaa ttcgccctta agctagcccc cagtggaaag acgcgcaggc   480

aaaacgcacc acgtgacgga gcgtgaccgc gcgccgagcc caaggtcggg caggaagagg   540

gcctatttcc catgattcct tcatatttgc atatacgata caaggctgtt agagagataa   600

ttagaattaa tttgactgta aacacaaaga tattagtaca aaatacgtga cgtagaaagt   660

aataatttct tgggtagttt gcagttttaa aattatgttt taaaatggac tatcatatgc   720

ttaccgtaac ttgaaagtat ttcgatttct tggctttata tatcttgtgg aaaggacgaa   780

acacccttac gctgagtact tcgattcaag agatcgaagt actcagcgta agtttttctc   840

gagttaaggg cgaattcccg attaggatct tcctagagca tggctacgta gataagtagc   900

atggcgggtt aatcattaac tacaaggaac ccctagtgat ggagttggcc actccctctc   960

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<211>  3920
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<220>
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       plasmid backbone, with AAV2 internal terminal repeats that flank
       expression cassette; referred to as AAV2/5 U6 lucRI-1b

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gcgtaaggct agcacacaaa aaaccaacac acagatctaa tgaaaataaa gatctttta c  1140

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         plasmid backbone, with AAV2 internal terminal repeats that flank
         expression cassette; referred to as AAV2/5 U6/U6 lucRIU6-3

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<212> DNA
<213> Artificial

<220>
<223> sequence for recombinant adeno-associated viral vector, including
      plasmid backbone, with AAV2 internal terminal repeats that flank
      expression cassette; referred to as AAV2/5 U6 lucRI-4(sense)

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<220>
<223>   sequence for recombinant adeno-associated viral vector, including
        plasmid backbone, with AAV2 internal terminal repeats that flank
        expression cassette; referred to as AAV2/5 U6 lucRI-4(antisense)

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